IN THE UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF DELAWARE

REGENXBIO INC. and THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA, Plaintiffs, v. SAREPTA THERAPEUTICS, INC. and SAREPTA THERAPEUTICS THREE, LLC, Defendants.	CASE NO. _____ JURY TRIAL DEMAND

COMPLAINT FOR PATENT INFRINGEMENT

REGENXBIO Inc. and The Trustees of the University of Pennsylvania (collectively "Plaintiffs"), by and through their undersigned attorneys, bring this action against Defendants Sarepta Therapeutics, Inc. ("Sarepta Inc.") and Sarepta Therapeutics Three, LLC ("Sarepta Three") (collectively, "Defendants" or "Sarepta") and hereby allege as follows:

NATURE OF ACTION

1. This is an action for infringement of United States Patent No. 10,526,617 ("the '617 Patent") instituted under the Patent Laws of the United States, 35 U.S.C. §§ 271 (a)-(c), arising from Defendants' manufacture and use of cultured host cell technology that is claimed in the '617 Patent to make recombinant adeno-associated virus ("AAV") gene therapy products that Defendants refers to as "SRP-9001 (AAVrh74.MHCK.micro-dystrophin)," which is used to treat Duchenne muscular dystrophy ("DMD"), and SRP-9003, which is used to treat Limb-girdle muscular dystrophy ("LGMD"), among other products. A true and accurate copy of the '617 Patent is attached as Exhibit A.

THE PARTIES

2. Plaintiff the Trustees of the University of Pennsylvania ("University") is a nonprofit corporation organized and existing under the laws of the State of Pennsylvania, with a place of business at 1 College Hall, Philadelphia, Pennsylvania 19104.

3. University is an institution of higher education and academic research and an academic medical center.

4. Plaintiff REGENXBIO Inc. (formerly ReGenX) ("REGENXBIO") is a company organized and existing under the laws of the State of Delaware, with a principal place of business at 9600 Blackwell Road, Suite 210, Rockville, MD 20850.

5. REGENXBIO is a clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO focuses on developing treatments for diseases with significant unmet needs, such as retinal, metabolic, and neurodegenerative diseases, using its patented NAV® Technology Platform, which includes, *inter alia*, the '617 Patent. In addition to its own programs in neurodegenerative and neuromuscular diseases, REGENXBIO has licensed many third parties under the NAV® patents in these disease areas.

6. Upon information and belief, Defendant Sarepta Inc. is a company organized and existing under the laws of the State of Delaware, with a principal place of business at 215 First St., Cambridge, MA 02142.

7. Upon information and belief, Defendant Sarepta Three is a wholly-owned subsidiary of Sarepta Inc. Upon further information and belief, Sarepta Three is a company organized and existing under the laws of the State of Delaware, with a principal place of business at 215 First St., Cambridge, MA 02142.

8. Upon information and belief, Sarepta Inc. is a biotechnology company in the business of, among other activities, developing products using AAV technology to treat diseases.

9. Upon information and belief, Sarepta Three is an entity involved in the commercialization and manufacture of products in collaboration with Sarepta Inc., including in the United States.

10. Upon information and belief, the cultured host cell technology of the '617 Patent is being used without a license for, *inter alia*, developing, manufacturing, and preparing to commercialize at least Sarepta's SRP-9001 and SRP-9003 products.

JURISDICTION AND VENUE

11. This is an action for patent infringement arising under the patent laws of the United States, 35 U.S.C. §§ 100 *et seq*., including §§ 271(a), 271(b), and 271(c). This Court has subject matter jurisdiction pursuant to 28 U.S.C. §§ 1331 and 1338(a).

12. Venue is proper in this district under 28 U.S.C. § 1400(b) and/or 28 U.S.C. §§ 1391(b) and (c).

13. This Court has personal jurisdiction over Sarepta Inc., as it is a corporation organized and existing under the laws of the State of Delaware, and since it has availed itself of the rights and benefits of Delaware law, it should reasonably anticipate being haled into court in this judicial district.

14. Court has personal jurisdiction over Sarepta Three as it is a corporation organized and existing under the laws of the State of Delaware, and since it has availed itself of the rights and benefits of Delaware law, it should reasonably anticipate being haled into court in this judicial district.

15. On information and belief, Sarepta Inc. and Sarepta Three have established, and will continue to maintain, minimum contacts with this judicial district such that the exercise of jurisdiction over Sarepta would not offend traditional notions of fair play and substantial justice.

FACTUAL BACKGROUND

Background Technology

16. Genetic changes — mutations or deletions in one's DNA — can cause serious disease or other metabolic dysfunctions that adversely impact health. People affected by such genetic changes face chronic disease and often require expensive medications to control their symptoms. Gene therapy offers a revolutionary alternative: a chance to treat the underlying cause of the symptoms by delivering a therapeutic gene, known as a "transgene," that corrects the course of disease and potentially provides lasting results from a single therapeutic dose.

17. Gene therapy can involve the use of a "vector" that packages and delivers a transgene into the body's cells. REGENXBIO has exclusive license rights to vectors invented at University, known as NAV® Vectors, composed of "capsid proteins" that package the transgene used to treat genetic defects or supply therapeutic factors such as antibodies to treat other serious conditions. Upon administration to a patient, the vectors deliver the transgenes to the nucleus of affected cells. Once there, transgenes serve as a genetic blueprint for new proteins that supply the function needed to treat or cure disease.

18. The claimed subject matter of the '617 Patent, discussed *infra,* is a cultured host cell containing a recombinant nucleic acid molecule encoding the capsid protein. It can be used in the process of creating the vectors (including certain NAV® Vectors) that deliver a transgene into cells in animal laboratory studies, or to deliver the transgene into cells in human subjects.

The vectors made using the claimed subject matter of the '617 Patent have unique properties, *e.g.*, an ability to target certain types of cells in the body.

The Patent-in-Suit

19. The '617 Patent is entitled "Method of Detecting and/or Identifying Adeno-Associated Virus (AAV) Sequences and Isolating Novel Sequences Identified Thereby," issued on January 7, 2020, names Guangping Gao, James M. Wilson, and Mauricio R. Alvira as inventors, and was assigned to University.

20. On May 31, 2002, University granted GlaxoSmithKline LLC ("GSK") an exclusive world-wide right and license, with the right to grant sublicenses, to various intellectual property rights, including the '617 Patent. Under that license agreement, GSK was also given the right to prosecute infringement claims, including for the '617 Patent.

21. On February 24, 2009, subject to certain limitations relating to the patents licensed to GSK, University granted ReGenX (now REGENXBIO) an exclusive world-wide right and license, with the right to grant sublicenses, to various intellectual property rights, including the '617 Patent. Under that license agreement, REGENXBIO was also given the right to prosecute infringement claims, including for the '617 Patent.

22. On March 6, 2009, GSK granted ReGenX (now REGENXBIO) an exclusive world-wide right and license, with the right to grant sublicenses, to various intellectual property rights, including the '617 Patent. Under that license agreement, REGENXBIO was also given the right to prosecute infringement claims, including for the '617 Patent.

23. GSK subsequently assigned to REGENXBIO any claims that GSK has arising from past, present, or future infringement of any claim in the '617 Patent by Sarepta.

24. Plaintiffs collectively have all substantial rights in and to the '617 Patent, including the right to assert any claims for past, present, and future infringement of the '617 Patent against Sarepta.

Count I
(Infringement of the '617 Patent)

25. Plaintiffs reallege paragraphs 1-24 as if fully set forth herein.

26. On information and belief, the manufacture and use of the patented cultured host cell claimed in the '617 Patent prior to the expiration of the '617 Patent for the production of SRP- 9001 constitutes direct infringement under 35 U.S.C. § 271(a), literally or under the doctrine of equivalents, of at least one claim of the '617 Patent.

27. On information and belief, each of Sarepta Inc. and Sarepta Three infringes, directly under 35 U.S.C. § 271(a), and/or indirectly under 35 U.S.C. §§ 271(b) or (c), at least claim 1 of the '617 Patent, which recites in full:

> 1. A cultured host cell containing a recombinant nucleic acid molecule encoding an AAV vp1 capsid protein having a sequence comprising amino acids 1 to 738 of SEQ ID NO: 81 (AAVrh.10) or a sequence at least 95% identical to the full length of amino acids 1 to 738 of SEQ ID NO: 81, wherein the recombinant nucleic acid molecule further comprises a heterologous non-AAV sequence.

28. Upon information and belief, SRP-9001 is produced by a process that includes making and using in the United States a cultured host cell containing a recombinant nucleic acid encoding an AAV vp1 capsid protein having a sequence at least 95% identical to the full length of amino acids 1 to 738 of SEQ ID NO: 81, wherein the recombinant nucleic acid molecule further comprises a heterologous non-AAV sequence.

29. Upon information and belief, SRP-9001 is a recombinant AAV gene therapy product that uses an AAVrh74 capsid protein to package and deliver a transgene. Upon

information and belief, the AAVrh74 vector has a vp1 capsid protein having a sequence at least 95% identical to the full length of amino acids 1 to 738 of SEQ ID NO: 81 (AAVrh.10). (*See, e.g.,* Ex. B ("The novel rAAV8-like serotype (rh.74) … is most similar to a related clade E virus rh.10 described by Gao and colleagues (99% amino acid identity)"); Ex. C (Nationwide Children's Hospital ("Nationwide") patent filing providing the amino acid sequence of the capsid protein of AAVrh74 at SEQ ID NO: 2); Ex. D (Nationwide Sequence Listing for WO 2013/078316); Ex. E (January 10, 2017, press release announcing agreement between Sarepta and Nationwide regarding a microdystrophin gene therapy program for treatment of DMD).)

30. Upon information and belief, SRP-9001 is produced by a process that includes making and using in the United States a cultured host cell containing a recombinant nucleic acid molecule encoding an AAVrh74 vp1 capsid protein and further comprising a heterologous non-AAV sequence. (*See, e.g.,* https://www.youtube.com/watch?v=kRV8NukPgaY; Ex. F (Sarepta-Aldevron Press Release).)

31. With regard to the entities that engage in such making and using, upon information and belief, that entity is either Sarepta Inc. and/or Sarepta Three, or an agent and/or contractual partner of either or both Sarepta Inc. and/or Sarepta Three to make and use the cultured host cell technology claimed in the '617 Patent. Thus, upon information and belief, Sarepta Inc. and/or Sarepta Three directly infringes at least claim 1 of the '617 Patent under 35 U.S.C. § 271(a) to the extent it makes and uses the cultured host cell technology claimed in the '617 Patent. Additionally or alternatively, to the extent that Sarepta Inc. and/or Sarepta Three instructs and/or contracts with others to make and use the cultured host cell technology claimed in the '617 Patent, Sarepta Inc. and/or Sarepta Three actively induces such infringement under 35 U.S.C. § 271(b). (*See, e.g.,* Ex. F (Sarepta-Aldevron Press Release); Ex. G (Sarepta-Brammer Bio Press Release);

Ex. H, excerpted Sarepta 10-K dated February 26, 2020[1] at 57 ("Sarepta [Three] will use Commercially Reasonable Efforts to Manufacture itself or have Manufactured on its behalf through its network of CMOs the Licensed Products [which include SRP-9001]….").) Moreover, to the extent Sarepta Inc. and/or Sarepta Three supplies others with components (such as plasmids encoding the AAVrh74 capsid protein), which have no substantially non-infringing uses, Sarepta contributes to such infringement under 35 U.S.C. § 271(c). (*Id.*)

32. Sarepta became aware of the '617 Patent no later than the date of filing of this Complaint. As a result, the use of the cultured host cell technology claimed in the '617 Patent for the production of SRP-9001 was made and will be made with full knowledge of the '617 Patent and without a reasonable basis for believing that Sarepta Inc. and/or Sarepta Three would not be liable for infringing or actively inducing or contributing to the infringement of the '617 Patent.

33. Sarepta has engaged in deliberate and willful behavior with knowledge of the '617 Patent and knew or should have known that its actions constituted direct and/or indirect infringement of the '617 Patent.

34. Although SRP-9001 requires Food and Drug Administration approval for marketing, the cultured host cells claimed in the '617 Patent, and used by Sarepta to produce SRP- 9001, do not.[2]

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[1] A full version of Sarepta's 10-K dated February 26, 2020 is available at https://investorrelations.sarepta.com/node/19251/html.

[2] Upon information and belief, SRP-9003, SRP-9004, SRP-9005, SRP-9006, MYO-201, Calpain 3, and GALGT2 are made using the same AAVrh74 capsid protein to package and deliver a transgene, and therefore Sarepta Inc. also directly and/or indirectly infringes the '617 Patent by making and using these gene therapy products for the same reasons. (Ex. H (excerpted Sarepta 10-K dated February 26, 2020, at 5 ("SRP-9003 utilizes the AAVrh.74 vector, the same vector used in SRP-9001."); Ex. J (SRP-9003 Clinical Update presentation); Ex. K (excerpted Bioinsights, The

35. On information and belief, SRP-9001 is currently in clinical development in the United States. (*See, e.g.*, Ex. H, excerpted Sarepta Inc. 10-K dated February 26, 2020, at 4.) On information and belief, Sarepta Three has entered into an agreement with F. Hoffman-La Roche Ltd. ("Roche") to develop and commercialize SRP-9001 outside the United States. On information and belief, both Sarepta and Roche are or will be seeking regulatory approval for SRP-9001 outside the United States.

36. Upon information and belief, SRP-9001 is made in the United States using a process that includes making and using cultured host cells that infringe at least claim 1 of the '617 Patent, for use inside and outside the United States. (*See, e.g.,* Ex. I, Sarepta 8-K filed December 23, 2019, at 2 (Sarepta "will manufacture and supply clinical and commercial supplies of SRP-9001 for itself and to [its licensee] Roche."); Ex. H at 10 (Sarepta "intend[s] to manufacture and supply all clinical and commercial supply of SRP-9001.")) Upon information and belief, Sarepta has made and/or will make a commercial supply of SRP-9001. (*See, e.g.,* Ex. H at 4 ("We plan to commence a trial evaluating SRP-9001 using commercial supply in the middle of 2020, pending regulatory feedback.")).

37. The making and using of the cultured host cell technology claimed in the '617 Patent for the production of SRP-9001 in violation of Plaintiffs' patent rights will cause harm to Plaintiffs for which damages are inadequate.

38. Unless and until Sarepta is enjoined from directly and/or indirectly infringing the '617 Patent, Plaintiffs will suffer substantial and irreparable harm for which there is no remedy at law.

Wonder Years-Gene Therapy Enters the Age of Adolescence) at 71); Ex. L (Sarepta Products Pipeline)).

JURY TRIAL DEMAND

39. Pursuant to Federal Rule of Civil Procedure 38(b), Plaintiffs hereby demand a trial by jury of all issues so triable.

PRAYER FOR RELIEF

Plaintiffs respectfully pray for the following relief:

a. That judgment be entered that Sarepta Inc. and/or Sarepta Three has infringed the '617 Patent by making and/or using the cultured host cell technology of the '617 Patent in the United States and/or by actively inducing and/or contributing to such infringement;

b. That an injunction be issued permanently enjoining Sarepta Inc. and/or Sarepta Three and its affiliates, officers, agents, employees, attorneys, and all persons in active concert or participation with any of them, from infringing the '617 Patent;

c. A preliminary and permanent injunction enjoining Sarepta Inc. and/or Sarepta Three, their respective officers, agents, servants, and employees, and those persons in active concert or participation with any of them, and their successors and assigns, from manufacturing or using the cultured host cell technology claimed in the '617 Patent or offering for sale or selling any resulting product prior to the expiration of the '617 Patent;

d. That Plaintiffs be awarded damages adequate to compensate them for the past, present, and/or future infringement of the '617 Patent by Sarepta Inc. and/or Sarepta Three, said damages being no less than a reasonable royalty together with any pre-judgment and post-judgment interest as allowed by law, costs, and other damages permitted by 35 U.S.C. § 284;

e. A judgment finding that Sarepta Inc.'s and/or Sarepta Three's infringement of the '617 Patent was deliberate and willful;

f. That this case be declared exceptional under 35 U.S.C. § 285, and that Plaintiffs be awarded reasonable attorneys' fees and costs;

g. That an accounting be performed to determine the damages to be awarded to Plaintiffs as a result of Sarepta Inc.'s and/or Sarepta Three's infringing activities, including an accounting for infringing conduct not presented at trial and an award of additional damages for any such infringing sales;

h. An award to Plaintiffs of costs and expenses they incur in prosecuting this action; and

i. That this Court award such other and further relief as it may deem just and proper.

Dated: September 15, 2020

FISH & RICHARDSON P.C.

By: */s/ Susan E. Morrison*

Susan E. Morrison (#4690)
Fish & Richardson P.C.
222 Delaware Avenue
17th Floor
Wilmington, DE 19801
morrison@fr.com
Tel: 302-652-5070

Attorneys for Plaintiffs

Brian Coggio
Jeremy T. Saks
Fish & Richardson P.C.
7 Times Square
20th Floor
New York, NY 10036

Kurt Glitzenstein
J. Peter Fasse
Fish & Richardson P.C.
1 Marina Park Drive
Boston, Massachusetts 02210